Top Medical Research Issues and Trends to Watch in 2017

Free Webinar
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fastercures.org
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A CENTER OF THE MILKEN INSTITUTE

10,000 DISEASES.
ONLY 500 TREATMENTS.
WE HAVE WORK TO DO.
Rx for Innovation

Recommendations for the New Administration
TOP TEN MEDICAL RESEARCH ISSUES AND TRENDS TO WATCH IN 2017

1. FDA: WHAT IS THE ROADMAP GOING FORWARD?
2. WHAT SIZE WOULD YOU LIKE: SMALL, MEDIUM OR BIG SCIENCE?
3. CLINICAL TRIAL INNOVATION AND REFORM: IT’S A BIG JOB BUT SOMEBODY’S GOTTA DO IT.
4. DOING SCIENCE IN THE REAL WORLD.
5. PATIENT-CENTRICITY: WHAT’S THE ROI?
6. MOVE OVER DIRECT-TO-CONSUMER ADS - IT’S TIME FOR DIRECT-TO-PATIENT R&D.
7. DIGITIZATION OF DISEASE, AND HEALTH.
8. DATA SHARING: WHERE THERE’S A WILL, THERE NEEDS TO BE A WAY.
9. EVOLUTION OF VALUE FRAMEWORKS.
10. BLOCKCHAIN COULD BE GOOD FOR YOUR HEALTH.
Innovation

• Investment in current **NIH priorities**
• **Clinical trial innovations** like adaptive and platform trials
• Changes at **FDA**
Data

• Is data sharing creating more “silos of excellence”?
• Researchers and FDA need to get on board with integrating real-world data and real-world evidence into health care and research
• Smart applications of computing power will help us better understand disease – and health
• Is blockchain hope or hype?
New Models

• **What’s the ROI** on patient engagement?
• Learn from direct-to-consumer leaders for “**direct-to-patient R&D**”
• **Patient Perspective Value Framework** aims to integrate patient perspectives in defining the value of new treatments
Anna Barker
Professor and Director,
Transformative Healthcare Networks, and Co-Director,
Complex Adaptive Systems Network
Arizona State University
What’s Trending in 2017: Clinical Trials

- “Smarter Trials” – Adaptive, learning systems and yes a “time machine”
- “Big data” comes to clinical trials
- Learning how to use “scruffy” data
- Algorithm (data)-driven vs. market or nonsense-driven trials
- Patient-centered trials – In the eye of the beholder
- The beginning of AI use in clinical trials
And 90% of these Data were Created in the Past 2 Years Across All Fields – 1% Analyzed!

“(Kryder’s law) Exponential growth of neuroimaging and genomics data, relative to increase of number of transistors per chip (Moore’s law)” (Image and Caption from Toga & Dinov, 2015).
Opportunities: Clinical Trials

• Building **learning systems**
• **Crowdsourcing knowledge** to build better trials (communities see the wisdom of real collaboration)
• **International collaboration** and participation – bringing diversity
• Building “**data lakes**” from clinical trials
• **Predictive biomarkers** – biomarker qualification to enable drug development
• Patients like “this one” – diseases like “this one” – **rare diseases/patients** may be our best opportunity
Threats: Clinical Trials

- The “standard” clinical trial – doing what we have always done
- No real focus on developing the **biomarkers needed to take time off the clock and reduce costs**
- **Lack of investment** to support new ideas
- **Reluctance to share data** – share knowledge (all sectors)
- **Too much reliance on “omics”** – not enough on patient data (we need to understand the natural history of disease)
- **Shortage of really innovative statisticians and data scientists** to conceive of new clinical trials models
- **Lack of leadership** from the industry – why take risks?
- **Fear** (FDA will never......Investors will never......patients will never......)
Who Needs a Seat at the Table: Clinical Trials

- **Groups of scientists** (basic, translational and clinical) to share the knowledge needed to **design trials that incorporate all that we know**!
- **Patients** (patients like “this one,” patients who want to become part of the process, patients who embrace innovation)
- **FDA** – the most innovative crowd around in this space
- **Biomarker scientists** – need to identified and developed
- **Next-generation statisticians** (age irrelevant) – thinking beyond the RCT
- **Data scientists** – using “scruffy” data, better analytics
- **Industry leaders** who “get it” and are fearless
- **Investors** who seem to embrace failure – must want better more predictable outcomes
- Increasingly this will be about **consortia/networks** who can build learning systems – not doing the same thing over and over and expecting different outcomes
The Forces Stressing The Biopharma Business Model

The useful patent life is shrinking

![Graph showing the useful patent life shrinking over time.](Source: QuintilesIMS)

Low-sales products dominate the industry

![Bar chart showing average annual sales in the first five years.](Source: QuintilesIMS)

R&D spending per NME keeps rising

![Graph showing R&D spent per NME.](Source: EvaluatePharma®)

Return to R&D for large-cap pharma keep declining

![Graph showing return to R&D for large-cap pharma.](Source: Deloitte, NYU)
The problem

Almost everything we’ve tried to improve our model has failed

- Biomarkers have only worked with highly penetrant mutations
- Efficiency drives have not reduced costs or complexity; or improved return on R&D
- Focus on blockbusters has not increased their number

Current pharma model depends increasingly upon questionable strategies for its viability

- Financial engineering (recurrent M&A; share buy-backs; tax inversions; extreme prices; coupon games)
- Orphan designations for the biggest drugs
- Patent fortresses around aging biological drugs

None of this produces innovation
This man wants us to change

Pharma is “getting away with murder”

We should listen, lest he concludes that he, and only he, can change drug R&D
Let’s leverage our opportunities

- Basic research turning out amazing discoveries
- New data-capture technologies that can slash the cost of collecting clinical data, and ease one of the most expensive bottlenecks in drug R&D
  - Gives us a chance to chip meaningfully at the $100 billion spend on clinical research, and $50 billion spend on discovery
- New infrastructure (e.g., million-patient cohorts; rapidly expanding knowledge in open/free databases) that enable smarter/faster research

Let’s stop what does not work; embrace what does; and change the economics of drug R&D
Who and how?

- Patients must be at the center of this transformation
  - Their diseases, their pain, their tissues, their data, their wallet

- Pushback must be overcome
  - From pharma, which is reluctant to try “unproven” pathways
  - From hospitals and CROs, which fear the loss of revenues
  - From clinicians who must embrace digital medicine

- FDA must offer continued guidance about expectations and requirements

- Payers must ease access and pay for performance

Can we build a coalition of the willing?

When do we start?
It's tough to make predictions, especially about the future.

(Yogi Berra)
PMI Cohort Program announces new name: the All of Us Research Program

We want to hear from you. Tell us what you think the PMI All of Us Program can achieve.

About the Precision Medicine Initiative®

Far too many diseases do not have proven preventions or treatments. To make a difference for the millions of Americans who suffer from them, we must gain better insights into the biological, environmental, and behavioral factors that drive these diseases. Precision medicine is an emerging approach for disease treatment and prevention that takes into account individual variability in environment, lifestyle and genes for each person.

On January 20, 2015, President Obama announced the Precision Medicine Initiative® (PMI) in his State of the Union address. As part of PMI, the NIH is leading the effort to build a national, large-scale research enterprise with one million or more volunteers to extend precision medicine to all diseases. The All of Us℠ Research Program...
The Precision Medicine Initiative – if not “precision medicine” – will be a reality by the end of 2017

- Enrolling participants across broad network of sites and via apps/web
- Providing data to researchers via cloud portal
- Returning data to participants

Related PMIs will be a reality as well

- NCI PMI / Moonshot
- California / other states
- China / other countries

Significant increase in available phenotypes!
ResearchKit Reaches a Crossroads - And Faces Some Tough Questions

Apple's groundbreaking digital health platform, launched in 2015 to revolutionize the clinical trials market, is facing competition from mHealth companies who say the platform has outgrown its usefulness.
Mobile research ecosystem emerges

First wave of ResearchKit now starting to yield peer reviewed science
- Stanford JAMA on MyHeartCounts
- Integration into clinical work

Second wave of platforms maturing
- ResearchStack for Android
- Private platforms trying to take market share

What will the tech stacks do?
These Startups Will Pay You For Your DNA

Patients usually don’t earn any money when they contribute to scientific discoveries and therapies. Some genetics startups want to change that.

Originally posted on Dec. 15, 2016, at 9:01 a.m.
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Stephanie M. Lee
BuzzFeed News Reporter

In The News Today

- President Donald Trump has commented on yesterday's massive Women's Marches asking, "why didn't these people vote?"
- Kellyanne Conway says White House press sec. Sean Spicer didn't lie about crowd size at Trump's inauguration. He gave "alternative facts."
- Members of the national security community reacted with shock after Trump attacked his critics while giving

Participant-centric experimentation

• **Business models** – subsidize data generation, mediate data sharing, return results / cash / data

• **Design models** – make complex decisions simpler, easier to understand and execute

• **Technical models** – make it easier to “ask” for data back / data transfer to 3rd party

• **Research models** – make it easier to sign up for studies / obtain informed consent
Q&A

Anna Barker
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Past Webinars

07/19/2016

**Countdown to a Cure: What’s Next for the Cancer Moonshot**

The Cancer Moonshot Summit, held June 29 in Washington, brought together hundreds of leaders across the health, academic, private industry, philanthropic and patient advocacy sectors under the national charge of doubling the rate of progress toward ending cancer as we know it. Join FasterCures for a free Webinar, Tuesday, July 19, from 11 a.m.

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06/01/2016

**Value Assessment 2.0: The Next Generation of Tools to Address the Patient Perspective**

We’ve managed to get patients more integrated into many areas of medical research, but a final frontier is putting patients into the discussion on value. FasterCures has long been engaged in work that strives to bring the patient perspective to the medical R&D process and, in partnership with Avalere, is committed to developing the first value framework that truly includes patient perspectives...

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Top Ten Medical Research Issues and Trends to Watch in 2017

by Margaret Anderson, Executive Director

As seen in the Huffington Post

"FDA needs strong allies and partners to ensure they are ready for the innovation and challenges ahead."

2016 will go down as a year that taught us to question our assumptions. The election of...
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